The CRISPR Way to Think about Duchenne's.

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Public Summary:
This article is a commentary that the New England Journal of Medicine invited me to write. The article explains and puts into perspective for NEJM readers a trio of recent papers that use a current method of genome editing to develop a gene therapy prototype in a mouse model of Duchenne's muscular dystrophy. In the article, I described what was done in the studies and interpreted the advantages and disadvantages of this approach. The purpose of the article was to give readers a good idea of how the CRISPR/Cas9 genome editing method was applied in these studies to correct a mutation in the dystrophin gene. I also covered how this new approach compares to two leading existing approaches and what hurdles the new approach would have to overcome to become a clinically meaningful therapy.

Scientific Abstract:
Duchenne's muscular dystrophy is caused by a loss-of-function mutation in DMD. Studies of the CRISPR-Cas9 method of excising the mutated region show efficacy in a mouse model of the disease.